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AUGUST 10, 2004

Division of Dockets Management (HFA-305)
Food and Drug Administration
Room 1061
5630 Fishers Lane
Rockville, MD 20852

Re: Genentech, Inc. Citizen Petition
FDA Docket No. 2004P-0171

Attached is the Statement of Policy prepared by the California Healthcare Institute on FDA Review of Biological Products.

David L. Gollaher, Ph.D.
President & CEO

2004P-0171

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California Healthcare Institute

Statement of Policy

on

FDA Review of Biological Products

This policy statement represents the position of the California Healthcare Institute (CHI) on the considerations that the Food and Drug Administration (FDA) must take into account in reviewing the safety and effectiveness of biological products. CHI members include more than 220 leading public and private research organizations and companies devoted to research and development at the frontiers of biotechnology. CHI's mission is to advocate responsible public policy that fosters medical innovation and promotes scientific discovery to advance the public health.

Background

Under current law, FDA reviews applications for the marketing of two types of pharmaceutical products under two separate federal statutes. New drugs are reviewed based on the submission of a new drug application (NDA) under the Federal Food, Drug, and Cosmetic Act (FD&C Act). Biological products are reviewed based on the submission of a biologics license application (BLA) under the Biological Products Act. In 1984, Congress amended the FD&C Act to establish a procedure for approval of generic versions of pioneer new drugs, after expiration of the patents and a fixed period of market exclusivity, based on a demonstration of bioequivalence to the pioneer new drug. Congress limited that procedure to new drugs marketed subject to NDAs and did not extend it to biological products marketed subject to BLAs. No comparable procedure for generic versions of biological products was established by Congress.

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As patents expire for pioneer biological products, questions have been raised about the regulatory requirements and standards that apply to FDA review of similar and related biological products that previously could not be marketed because they would have violated applicable patents. These products are often referred to as generic or follow-on biological products. Their regulatory status raises important scientific, legal, and public policy issues, the resolution of which will vitally affect the future of the biotechnology industry and the public health.

Incentives for Scientific Innovation

The future improvement of healthcare throughout the world depends upon preservation of strong incentives for the scientific innovation that will be essential to the research and development of new biological products to reduce the burden of illness and disease. The potential for lifesaving and life-enhancing biological products was made feasible by the discovery of the structure of DNA by Watson and Crick fifty years ago. The biotechnology tools to turn this basic science into revolutionary new biological products began with the development of recombinant DNA technology in the mid-1970s, and has been carried forward with new scientific methodologies ever since. The research and development necessary to discover, formulate, test, and obtain FDA approval for marketing these important new biological products has been carried on in government research laboratories like NIH, private scientific centers like Scripps and Salk, small startup biotechnology companies funded by venture capital, and large pharmaceutical concerns funded by the profits of existing products. Dozens of innovative new biological products have already been marketed, and hundreds are currently in various stages of development. If even a portion of these products that are currently under development eventually reach the market, it will represent the greatest medical revolution in human history.

The progress that has been made to date, and the promise of even greater progress in the future, rest upon incentives for innovation. It requires the continued close cooperation of government, academia, and private enterprise. Public policy must therefore recognize and reflect

sufficiently strong and consistent incentives to assure that there is adequate reward for successful research and development of important new biological products. If those incentives falter or are weakened, our continued progress in public health will be correspondingly threatened and the full promise of the biotechnology revolution will not be achieved.

Assurance of the Safety and Effectiveness of Biological Products

Americans expect and deserve that all biological products will be reviewed and approved by FDA for both safety and effectiveness. The statute under which biological products are regulated -- the Biological Products Act -- represented the first time in human history that any product was required to obtain a governmental license before the product could be lawfully marketed. It is essential that FDA regulations reflect state-of-the-art science and that the requirements for all biological products are commensurate with what is needed to assure safety and effectiveness for patients.

The principles established by the Biological Products Act apply with equal force today. No biological product can be expected to be absolutely safe or absolutely effective. Each product must be evaluated on the basis of benefit and risk. But we can and must expect that FDA, acting as an independent and neutral scientific judge, will review all of the pertinent data and information relating to each individual product and will make a determination that the safety and effectiveness have or have not adequately been established.

Protection of Confidential Commercial Information

The investment needed to finance the research, development, and approval of a biological product today is staggering. According to published studies, the average cost of a new marketed biological product has risen from less than $150 million in 1970 to more than $1 billion today. The time from discovery to product approval has more than doubled. Our nation's public and private funding of pharmaceutical research has increased exponentially. We undertake this massive

research not just for the sake of scientific discovery, but because it is the only way we can hope to alleviate illness and save lives.

The American free enterprise system, which has produced the most successful medical research in the history of the world, depends upon two fundamental principles. First, all relevant data and information pertaining to the safety and effectiveness of a biological product must be submitted to and evaluated by FDA as part of the regulatory approval system. Second, all of that information constitutes trade secrets and confidential commercial information that are explicitly exempt from public disclosure under the Freedom of Information Act and the public disclosure of which is a crime under the Federal Trade Secrets Act and a violation of the Takings Clause of the Fifth Amendment to the United States Constitution. FDA has no authority to approve one individual's BLA on the basis of data or information contained in another individual's BLA. It has been recognized since 1902 that each biological product is separate and distinct, and must be evaluated on the basis of its own safety and effectiveness data.

This policy is grounded on sound science and fundamental economic principles. No two biological products, made in different establishments, can be assured to be identical. For more than a hundred years, government regulatory policy has recognized that the inevitable differences among biological products produced in different establishments using diverse manufacturing methods preclude reliance upon one product to approve a second product. From an economic standpoint, if one individual could use another's data to justify the marketing of a generic version it would undermine the financial basis on which the biotechnology industry is founded. The investment that is so vital to the continuing growth of small biotechnology companies, which pursue some of the most important cutting-edge scientific research of our day, is premised on the understanding that trade secrets and confidential commercial information will be respected and protected by the government and cannot be used for the approval of generic biological products.

A Democratic Process

Our current law governing the regulation of biological products has been developed in the way that all important public policy issues are developed in a democratic society -- through vigorous public debate, probing congressional hearings, and ultimately enactment of a national statute. Under the Biological Products Act, biological products have now been regulated in the same way for more than a hundred years. If there are to be any changes, they should be adopted in the same way that the current policy was adopted, through new legislation. Generic biological products cannot be approved on the basis of data or information contained in the BLA for the pioneer product unless and until Congress changes the law. FDA must respect the same principle for the very few biological products that have been approved under NDAs.

These matters are of fundamental importance to the public health. Our country established a system of regulatory controls over biological products that have assured the safety and effectiveness of these products and provided adequate incentives for continually increased investment in research and development for the new products of the future. Before any steps are taken to change or dismantle this remarkably productive construct, there must be a broad public consensus and adequate assurance that the public health will not be harmed.

Sincerely,

David L. Gollaher, Ph.D.
President & CEO